

Title (en)

METHODS AND COMPOSITIONS FOR TREATING THALASSEMIA OR SICKLE CELL DISEASE

Title (de)

VERFAHREN UND ZUSAMMENSETZUNGEN ZUR BEHANDLUNG VON THALASSÄMIE UND SICHELZELLANÄMIE

Title (fr)

PROCÉDÉS ET COMPOSITIONS POUR LE TRAITEMENT DE LA THALASSÉMIE OU DE LA DRÉPANOCYTOSE

Publication

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Application

EP 20831340 A 20200629

Priority

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- US 2020040199 W 20200629

Abstract (en)

[origin: WO2020264532A1] Provided herein are methods and compositions for treating genetic blood cell diseases, e.g. sickle cell disease and thalassemia, by correcting genetic mutation or inserting exogenous globin gene using CRISPR/Cas system.

IPC 8 full level

C12N 15/90 (2006.01); **A61K 35/28** (2015.01); **A61K 48/00** (2006.01); **C12N 5/0789** (2010.01); **C12N 9/22** (2006.01); **C12N 15/11** (2006.01); **C12N 15/52** (2006.01)

CPC (source: EP US)

A61K 35/28 (2013.01 - EP US); **C07K 14/805** (2013.01 - EP US); **C12N 9/22** (2013.01 - EP US); **C12N 15/102** (2013.01 - EP); **C12N 15/11** (2013.01 - US); **C12N 15/113** (2013.01 - EP); **C12N 15/907** (2013.01 - EP US); **A61K 38/00** (2013.01 - EP US); **A61K 48/005** (2013.01 - EP); **C12N 2310/20** (2017.05 - EP US); **C12N 2750/14143** (2013.01 - EP)

Citation (search report)

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- [A] WO 2016154579 A2 20160929 - EDITAS MEDICINE INC [US]
- [A] SHUQIAN XU ET AL: "Editing aberrant splice sites efficiently restores b-globin expression in b-thalassemia", BLOOD, vol. 133, no. 21, 31 January 2019 (2019-01-31), pages 2255 - 2262, XP055689244, DOI: 10.1182/blood-2019-01-895094
- See also references of WO 2020264532A1

Designated contracting state (EPC)

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